Understanding through observation in natural history studies of the disease. Example: Verhage, CHOP, registry. Developing Clinical Trial Readiness

Phase I/II Clinical trials evaluate the safety and preliminary effectiveness of a treatment, and the amount of the medication needed in a small group of patients. Examples: Cornell 4PB drug trial

Phase III Clinical trials which include a larger group of patients to further establish the effectiveness and safety of the drug. FDA and EMA reviews findings from research and clinical trial to approve treatment.

Ongoing studies for long-term monitoring of patients receiving therapies. Use insights gained in future therapy development.

We are stronger together!